International Patent Application No. PCT/DE00/00244
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New Patent Claims

1. Method for inhibiting the expression of a given target gene in a cell in vitro, where an oligoribonucleotide with double-stranded structure (dsRNA) formed by two separate RNA single strands is introduced into the cell, where one strand of the dsRNA has a region which is complementary to the target gene,
characterized in that
the complementary region has less than 25 successive nucleotide pairs.

2. Method according to claim 1, where the dsRNA is enclosed by micellar structures, preferably by liposomes.

3. Method according to either of the preceding claims, where the dsRNA is enclosed by natural viral capsids or by chemically or enzymatically produced artificial capsids or structures derived therefrom.

4. Method according to one of the preceding claims, where the target gene is expressed in eukaryotic cells.

5. Method according to one of the preceding claims, where the target gene is selected from the following group: oncogene, cytokin gene, Id-protein gene, development gene, prion gene.

6. Method according to one of the preceding claims, where the target gene is expressed in pathogenic organisms, preferably in plasmodia.

7. Method according to one of the preceding claims, where the target gene is part of a virus or viroid.

8. Method according to claim 7, where the virus is a virus or viroid which is pathogenic for humans.

9. Method according to claim 7, where the virus or viroid is a virus or viroid which is pathogenic for animals or phytopathogenic.

10. Method according to one of the preceding claims, where segments of the dsRNA are in double-stranded form.

11. Method according to one of the preceding claims, where the ends of the dsRNA are modified in order to counteract degradation in the cell or dissociation into the single strands.

12. Method according to one of the preceding claims, where the cohesion of the double-stranded structure, which is caused by the complementary nucleotide pairs, is increased by at least one, preferably two, further chemical linkage(s).

13. Method according to one of the preceding claims, where the chemical linkage is formed by a covalent or ionic bond, a hydrogen bond, hydrophobic interactions, preferably van-der-Waals or stacking interactions, or by metal-ion coordination.

14. Method according to one of the preceding claims, where the chemical linkage is generated at at least one, preferably both, ends of the double-stranded structure.

15. Method according to one of the preceding claims, where the chemical linkage is formed by means of one or more compound groups, the compound groups preferably being poly(oxyphosphinicooxy-1,3-propanediol) and/or polyethylene glycol chains.

16. Method according to one of the preceding claims, where the chemical linkage is formed by purine analogs used in the double-stranded structure in place of purines.

17. Method according to one of the preceding claims, where the chemical linkage is formed by azabenzene units introduced into the double-stranded structure.

18. Method according to one of the preceding claims, where the chemical linkage is formed by branched nucleotide analogs used in the double-stranded structure in place of nucleotides.

19. Method according to one of the preceding claims, where at least one of the following groups is used for generating the chemical linkage: methylene blue; bifunctional groups, preferably bis(2-chloroethyl)amine; N-acetyl-N'-(p-glyoxyl-benzoyl)cystamine; 4-thiouracil; psoralene.

20. Method according to one of the preceding claims, where the chemical linkage is formed by thiophosphoryl groups provided at the ends of the double-stranded structure.

21. Method according to one of the preceding claims, where the chemical linkage at the ends of the double-stranded structure is formed by triple-helix bonds.

22. Method according to one of the preceding claims, where at least one 2'-hydroxyl group of the nucleotides of the dsRNA in the double-stranded structure is replaced by a chemical group, preferably a 2'-amino or a 2'-methyl group.

23. Method according to one of the preceding claims, where at least one nucleotide in at least one strand of the double-stranded structure is a locked nucleotide with a sugar ring which is chemically modified, preferably by a 2'-O, 4'-C-methylene bridge.

24. Method according to one of the preceding claims, where the dsRNA is bound to, associated with or surrounded by, at least one viral coat protein which originates from a virus, is derived therefrom or has been prepared synthetically.

25. Method according to one of the preceding claims, where the coat protein is derived from polyomavirus.

26. Method according to one of the preceding claims, where the coat protein contains the polyomavirus virus protein 1 (VP1) and/or virus protein 2 (VP2).

27. Method according to one of the preceding claims, where, when a capsid or capsid-type structure is formed from the coat protein, one side faces the interior of the capsid or capsid-type structure.

28. Method according to one of the preceding claims, where one strand of the dsRNA is complementary to the primary or processed RNA transcript of the target gene.

29. Method according to one of the preceding claims, where the cell is a vertebrate cell or a human cell.

30. Method according to one of the preceding claims, where at least two dsRNAs which differ from each other are introduced into the cell, where at least segments of one strand of each dsRNA are complementary to in each case one of at least two different target genes.

31. Method according to one of the preceding claims, where one of the target genes is the PKR gene.

32. Medicament with at least one oligoribonucleotide with double-stranded structure (dsRNA) formed by two separate RNA single strands for inhibiting the expression of a given target gene, where one strand of the dsRNA has a region which is complementary to the target gene, characterized in that the complementary region has less than 25 successive nucleotide pairs.

33. Medicament according to claim 32, where the dsRNA is enclosed by micellar structures, preferably by liposomes.

34. Medicament according to either of claims 32 or 33, where the dsRNA is enclosed by natural viral capsids or by chemically or enzymatically produced artificial capsids or structures derived therefrom.

35. Medicament according to one of claims 32 to 34, where the target gene can be expressed in eukaryotic cells.

36. Medicament according to one of claims 32 to 35, where the target gene is selected from the following group: oncogene, cytokin gene, Id-protein gene, development gene, prion gene.

37. Medicament according to one of claims 32 to 36, where the target gene can be expressed in pathogenic organisms, preferably in plasmodia.

38. Medicament according to one of claims 32 to 37, where the target gene is part of a virus or viroid.

39. Medicament according to claim 38, where the virus is a virus or viroid which is pathogenic for humans.

40. Medicament according to claim 38, where the virus or viroid is a virus or viroid which is pathogenic for animals.

41. Medicament according to one of claims 32 to 40, where segments of the dsRNA are in double-stranded form.

42. Medicament according to one of claims 32 to 40, where the ends of the dsRNA are modified in order to counteract degradation in the cell or dissociation into the single strands.

43. Medicament according to one of claims 32 to 42, where the cohesion of the double-stranded structure, which is caused by the complementary nucleotide pairs, is increased by at least one, preferably two, further chemical linkage(s).

44. Medicament according to one of claims 32 to 43, where the chemical linkage is formed by a covalent or ionic bond, a hydrogen bond, hydrophobic interactions, preferably van-der-Waals or stacking interactions, or by metal-ion coordination.

45. Medicament according to one of claims 32 to 44, where the chemical linkage is generated at at least one, preferably both, ends of the double-stranded structure.

46. Medicament according to one of claims 32 to 45, where the chemical linkage is formed by means of one or more compound groups, the compound groups preferably being poly(oxyphosphinicooxy-1,3-propanediol) and/or polyethylene glycol chains.

47. Medicament according to one of claims 32 to 46, where the chemical linkage is formed by purine analogs used in the double-stranded structure in place of purines.

48. Medicament according to one of claims 32 to 47, where the chemical linkage is formed by azabenzene units inserted into the double-stranded structure.

49. Medicament according to one of claims 32 to 48, where the chemical linkage is formed by branched nucleotide analogs used in the double-stranded structure in place of nucleotides.

50. Medicament according to one of claims 32 to 49, where at least one of the following groups is used for generating the chemical linkage: methylene blue; bifunctional groups, preferably bis(2-chloroethyl)amine; N-acetyl-N'-(p-glyoxylbenzoyl)-cystamine; 4-thiouracil; psoralene.

51. Medicament according to one of claims 32 to 50, where the chemical linkage is formed by thiophosphoryl groups provided at the ends of the double-stranded structure.

52. Medicament according to one of claims 32 to 51, where the chemical linkage are [sic] triple-helix bonds provided at the ends of the double-stranded structure.

53. Medicament according to one of claims 32 to 52, where at least one 2'-hydroxyl group of the nucleotides of the dsRNA in the double-stranded structure is replaced by a chemical group, preferably a 2'-amino or a 2'-methyl group.

54. Medicament according to one of claims 32 to 53, where at least one nucleotide in at least one strand of the double-stranded structure is a locked nucleotide with a sugar ring which is chemically modified, preferably by a 2'-O, 4'-C-methylene bridge.

55. Medicament according to one of claims 32 to 54, where the dsRNA is bound to, associated with or surrounded by, at least one viral coat protein which originates from a virus, is derived therefrom or has been prepared synthetically.

56. Medicament according to one of claims 32 to 55, where the coat protein is derived from the polyomavirus.

57. Medicament according to one of claims 32 to 56, where the coat protein contains the polyomavirus virus protein 1 (VP1) and/or virus protein 2 (VP2).

58. Medicament according to one of claims 32 to 57, where, when a capsid or capsid-type structure is formed from the coat protein, one side faces the interior of the capsid or capsid-type structure.

59. Medicament according to one of claims 32 to 58, where one strand of the dsRNA is complementary to the primary or processed RNA transcript of the target gene.

60. Medicament according to one of claims 32 to 59, where the cell is a vertebrate cell or a human cell.

61. Medicament according to one of claims 32 to 60, where at least two dsRNAs which differ from each other are contained in the medicament, where at least segments of one strand of each dsRNA are complementary to in each case one of at least two different target genes.

62. Medicament according to claim 61, where one of the target genes is the PKR gene.

63. Active ingredient with at least one oligoribonucleotide with double-stranded structure (dsRNA) formed by two separate RNA single strands for inhibiting the expression of a given target gene, where one strand of the dsRNA has a region which is complementary to the target gene, and where the target gene is part of a phytopathogenic virus or viroid,
characterized in that
the complementary region has less than 25 successive nucleotide pairs.

64. Active ingredient according to claim 63, where the target gene can be expressed in eukaryotic cells.

65. Active ingredient according to claim 63 or 64, where segments of the dsRNA are in double-stranded form.

66. Active ingredient according to one of claims 63 to 65, where the ends of the dsRNA are modified in order to counteract degradation in the cell or dissociation into the single strands.

67. Active ingredient according to one of claims 63 to 66, where the cohesion of the double-stranded structure, which is caused by the complementary nucleotide pairs, is increased by at least one, preferably two, further chemical linkage(s).

68. Active ingredient according to one of claims 63 to 67, where the chemical linkage is formed by a covalent or ionic bond, a hydrogen bond, hydrophobic interactions, preferably van-der-Waals or stacking interactions, or by metal-ion coordination.

69. Active ingredient according to one of claims 63 to 68, where the chemical linkage is generated at at least one, preferably both, ends of the double-stranded structure.

70. Active ingredient according to one of claims 63 to 69, where the chemical linkage is formed by means of one or more compound groups, the compound groups preferably being poly(oxyphosphinicooxy-1,3-propanediol) and/or polyethylene glycol chains.

71. Active ingredient according to one of claims 63 to 70, where the chemical linkage is formed by purine analogs used in the double-stranded structure in place of purines.

72. Active ingredient according to one of claims 63 to 71, where the chemical linkage is formed by azabenzene units inserted into the double-stranded structure.

73. Active ingredient according to one of claims 63 to 72, where the chemical linkage is formed by branched nucleotide analogs used in the double-stranded structure in place of nucleotides.

74. Active ingredient according to one of claims 63 to 73, where at least one of the following groups is used for generating the chemical linkage: methylene blue; bifunctional groups, preferably bis(2-chloroethyl)amine; N-acetyl-N'-(p-glyoxyl-benzoyl)cystamine; 4-thiouracil; psoralene.

75. Active ingredient according to one of claims 63 to 74, where the chemical linkage is formed by thiophosphoryl groups provided at the ends of the double-stranded structure.

76. Active ingredient according to one of claims 63 to 75, where the chemical linkage are triple-helix bonds provided at the ends of the double-stranded structure.

77. Active ingredient according to one of claims 63 to 76, where at least one 2'-hydroxyl group of the nucleotides of the dsRNA in the double-stranded structure is replaced by a chemical group, preferably a 2'-amino or a 2'-methyl group.

78. Active ingredient according to one of claims 63 to 77, where at least one nucleotides at least one strand of the double-stranded structure is a locked nucleotide with a sugar ring which is chemically modified, preferably by a 2'-O, 4'-C-methylene bridge.

79. Active ingredient according to one of claims 63 to 78, where one strand of the dsRNA is complementary to the primary or processed RNA transcript of the target gene.

80. Active ingredient according to one of claims 63 to 79, where at least two dsRNAs which differ from each other are contained in the active ingredient, where at least segments of one strand of each dsRNA are complementary to in each case one of at least two different target genes.

81. Use of an oligoribonucleotide with double-stranded structure (dsRNA) formed by two separate RNA single strands or preparing a medicament or active ingredient for inhibiting the expression of a given target gene, where one strand of the dsRNA has a region which is complementary to the target gene,
characterized in that
the complementary region has less than 25 successive nucleotide pairs.

82. Use according to claim 81, where the dsRNA is enclosed by micellar structures, preferably by liposomes.

83. Use according to either of claims 81 or 82, where the dsRNA is enclosed by natural viral capsids or by chemically or enzymatically produced artificial capsids or structures derived therefrom.

84. Use according to one of claims 81 to 83, where the target gene can be expressed in eukaryotic cells.

85. Use according to one of claims 81 to 84, where the target gene is selected from the following group: oncogene, cytokin gene, Id-protein gene, development gene, prion gene.

86. Use according to one of claims 81 to 85, where the target gene can be expressed in pathogenic organisms, preferably in plasmodia.

87. Use according to one of claims 81 to 86, where the target gene is part of a virus or viroid.

88. Use according to claim 87, where the virus is a virus or viroid which is pathogenic for humans.

89. Use according to claim 87, where the virus or viroid is a virus or viroid which is pathogenic for animals or phytopathogenic.

90. Use according to one of claims 81 to 89, where segments of the dsRNA are in double-stranded form.

91. Use according to one of claims 81 to 90, where the ends of the dsRNA are modified in order to counteract degradation in the cell or dissociation into the single strands.

92. Use according to one of claims 81 to 91, where the cohesion of the double-stranded structure, which is caused by the complementary nucleotide pairs, is increased by at least one, preferably two, further chemical linkage(s).

93. Use according to one of claims 81 to 92, where the chemical linkage is formed by a covalent or ionic bond, a hydrogen bond, hydrophobic interactions, preferably van-der-Waals or stacking interactions, or by metal-ion coordination.

94. Use according to one of claims 81 to 93, where the chemical linkage is generated at at least one, preferably both, ends of the double-stranded structure.

95. Use according to one of claims 81 to 94, where the chemical linkage is formed by means of one or more compound groups, the compound groups preferably being poly(oxyphosphinicooxy-1,3-propanediol) and/or polyethylene glycol chains.

96. Use according to one of claims 81 to 95, where the chemical linkage is formed by purine analogs used in the double-stranded structure in place of purines.

97. Use according to one of claims 81 to 96, where the chemical linkage is formed by azabenzene units introduced into the double-stranded structure.

98. Use according to one of claims 81 to 97, where the chemical linkage is formed by branched nucleotide analogs used in the double-stranded structure in place of nucleotides.

99. Use according to one of claims 81 to 98, where at least one of the following groups is used for generating the chemical linkage: methylene blue; bifunctional groups, preferably bis(2-chloroethyl)amine; N-acetyl-N'-(p-glyoxylbenzoyl)-cystamine; 4-thiouracil; psoralene.

100. Use according to one of claims 81 to 99, where the chemical linkage is formed by thiophosphoryl groups attached to the ends of the double-stranded structure.

101. Use according to one of claims 81 to 100, where the chemical linkage at the ends of the double-stranded structure is formed by triple-helix bonds.

102. Use according to one of claims 81 to 101, where at least one 2'-hydroxyl group of the nucleotides of the dsRNA in the double-stranded structure is replaced by a chemical group, preferably a 2'-amino or a 2'-methyl group.

103. Use according to one of claims 81 to 102, where at least one nucleotide in at least one strand of the double-stranded structure is a locked nucleotide with a sugar ring which is chemically modified, preferably by a 2'-O, 4'-C-methylene bridge.

104. Use according to one of claims 81 to 103, where the dsRNA is bound to, associated with or surrounded by, at least one viral coat protein which originates from a virus, is derived therefrom or has been prepared synthetically.

105. Use according to one of claims 81 to 104, where the coat protein is derived from polyomavirus.

106. Use according to one of claims 81 to 105, where the coat protein contains the polyomavirus virus protein 1 (VP1) and/or virus protein 2 (VP2).

107. Use according to one of claims 81 to 106, where, when a capsid or capsid-type structure is formed

from the coat protein, one side faces the interior of the capsid or capsid-type structure.

108. Use according to one of claims 81 to 107, where one strand of the dsRNA is complementary to the primary or processed RNA transcript of the target gene.

109. Use according to one of claims 81 to 108, where the cell is a vertebrate cell or a human cell.

110. Use according to one of claims 81 to 109, where at least two dsRNAs which differ from each other are used, where at least segments of one strand of each dsRNA are complementary to in each case one of at least two different target genes.

111. Use according to claim 110, where one of the target genes is the PKR gene.

112. Use according to one of claims 81 to 111, where the medicament is injectable into the bloodstream or into the interstitium of the organism to undergo therapy.

113. Use according to one of claims 81 to 112, where the dsRNA is taken up into bacteria or microorganisms.

114. Use of a vector for coding at least one oligoribonucleotide with double-stranded structure (dsRNA) formed by two separate RNA single strands for preparing a medicament or active ingredient for inhibiting the expression of a given target gene, where one strand of the dsRNA has a region which is complementary to the target gene, characterized in that

the complementary region has less than 25 successive nucleotide pairs.

115. Use according to claim 114, where the target gene can be expressed in eukaryotic cells.

116. Use according to claim 114 or 115, where the target gene is selected from the following group: oncogene, cytokin gene, Id-protein gene, development gene, prion gene.

117. Use according to one of claims 114 to 116, where the target gene can be expressed in pathogenic organisms, preferably in plasmodia.

118. Use according to one of claims 114 to 117, where the target gene is part of a virus or viroid.

119. Use according to claim 118, where the virus is a virus or viroid which is pathogenic for humans.

120. Use according to claim 118, where the virus or viroid is a virus or viroid which is pathogenic for animals or phytopathogenic.

121. Use according to one of claims 114 to 120, where segments of the dsRNA are in double-stranded form.

122. Use according to one of claims 114 to 121, where one strand of the dsRNA is complementary to the primary or processed RNA transcript of the target gene.

123. Use according to one of claims 114 to 122, where the cell is a vertebrate cell or a human cell.

124. Use according to one of claims 114 to 123, where at least two dsRNAs which differ from each other

are used, where at least segments of one strand of each dsRNA are complementary to in each case one of at least two different target genes.

125. Use according to claim 125, where one of the target genes is the PKR gene.